From: mailsvc@alpha.ddm.uci.edu on behalf of Virginia Kimonis, M.D., Chief, UCI Division of Genetics & Metabolism
Subject: Advances in Neuromuscular Diseases, Feb. 7 at 6 p.m.
Date: Thursday, February 02, 2012 2:21:05 PM

UC Irvine/CHOC Division of Genetics, MDA ALS and Neuromuscular Center, and ICTS Invite You to Attend a Dinner Discussion and Team Building Conference

Advances in Neuromuscular Diseases: Investigational Treatments and Research Initiatives

Tuesday, Feb. 7
6-7:30 p.m.
Dinner will be provided

Children's Hospital of Orange County (CHOC)
CHOC Clinic/Research Building
Conference Room C and D, 2nd Floor
455 S. Main St., Orange, CA 92868
(Please save parking ticket for validation)
http://www.choc.org/userfiles/file/choccampus.pdf

Barry J. Byrne, M.D., Ph.D.
Professor and Associate Chair of Research, Department of Pediatrics and Microbiology and Molecular Genetics
Virginia Root Sutherland Professor of Cardiology
Director, Powell Gene Therapy Center
Medical Director, Congenital Heart Center
University of Florida, College of Medicine

Tahseen Mozaffar, M.D.
Director, UC Irvine-MDA ALS and Neuromuscular Center

RSVP by Feb. 3 to Marie Wencel at mwencel@uci.edu or 949.824.0521

Supported by an educational grant from Genzyme Corporation

Barry J. Byrne, M.D., Ph.D.
Dr. Byrne is internationally recognized for his work in the areas of cardiomyopathy, transplantation and genetic therapy. His laboratory is actively involved in developing new genetic therapies for cardiovascular disease. In the area of cardiomyopathy, his lab is studying gene replacement in an autosomal recessive form of fatal cardiomyopathy in children. The disease is the prototype of lysosomal storage disorders

leading to skeletal and cardiac muscle weakness. The lab has used AAV vectors to achieve sustained correction of the gene deficiency and correction of the phenotype in natural and transgenic mouse models of the disease. The current therapy is currently being proposed for human clinical trials. Similar therapies are being used to combat cardiac transplantation rejection. Secondly, the lab is investigating the ability of mesenchymal stem cells to undergo myocardial specification for the purpose of tissue repair in the heart. Finally, several projects are focused on the use of AAV vectors injected into striated muscle to achieve sustained release of therapeutic proteins, including thrombolytic factors and coagulation factors. These projects are currently funded by the National Institutes of Health (NIH) and the American Heart Association (AHA).